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ALLOGENEIC TRANSPLANTATION FOR WISKOTT ALDRICH SYNDROME USING A REDUCED INTENSITY CONDITIONING REGIMEN

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Background: Allogeneic stem cell transplantation (SCT) is curative for Wiskott Aldrich syndrome (WAS), an X-linked disorder with thrombocytopenia, immune deficiency, and susceptibility to malignancy/autoimmunity. Standard myeloablative conditioning regimen for SCT often result in lasting toxicities. Hence, we used reduced intensity conditioning (RIT) in 2 patients whose disease manifestations included infections and hemorrhage.

Methods: The 2 males (R1 and R2), 7 and 8 months old, underwent SCT with bone marrow from a HLA identical sibling and 10/10 allele matched unrelated donor respectively. Conditioning consisted of 23 and 28 mg of Campath-1H (targeted dose was 33 mg) on days -22 and -19, fludarabine (1.0 mg/kg/day) on day -8 to -4, and melphalan (4.7 mg/kg) on day -3. GVHD prophylaxis included cyclosporine, short course methotrexate, and methylprednisolone. GCSF was administered until neutrophil engraftment.

Results: Campath-1H was associated with vasculitic edema and blisters in R1 resulting in lowered dosing. R2 received a lower dose following an unrelated aspiration episode. Neutrophils engrafted (ANC ≥500/dl) on days 15 and 12; platelets engrafted (≥20,000/dl) on days 70 and 13 respectively. Neither developed ≥grade 2 GVHD or severe organ toxicity. R1 is off immunosuppression and R2 is on a cyclosporine taper. By molecular analysis 9 months post SCT, R1 has 81% lymphoid and 22% myeloid; R2 has 100% lymphoid and 65% myeloid donor chimerism that is stable post immunosuppression taper. R2 developed autoimmune hemolytic anemia that responded to rituximab therapy. Neither had bleeding complications. Lymphocyte subpopulations and function were recovering 6 months post SCT.

Conclusions: This reduced intensity conditioning achieved mixed donor chimerism in 2 patients with WAS, resulting in transfusion independence and amelioration of bleeding complications. SCT was not associated with significant organ toxicity or GVHD. Monitoring for durability of engraftment and other transplant related complications is in progress. A reduced intensity transplant that allows stable donor engraftment and limits chemotherapy may spare young patients toxicities of standard conditioning and merits further investigation.

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CHARACTERISTICS OF THE PEDIATRIC BLOOD AND MARROW TRANSPLANT ADVANCED PRACTICE NURSE

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Purpose: This survey was designed to identify the demographics and professional responsibilities of a Pediatric Advanced Practice Nurse (APN) in the field of Blood & Marrow Transplantation (BMT) within the Pediatric Blood & Marrow Transplant Consortium (PBMTC).

Methods: A survey was sent to nurses within institutions that participate in the PBMTC. The survey was designed to collect information about personal and institutional demographics, transplant specific responsibilities and procedures.

Results: Thirty-one surveys were returned representing responses from various institutions. Demographic characteristics of the APN are reported in Table 1. All APN's report performing both autologus and allogeneic transplants. Eight-three percent are individual pediatric transplant programs with 75% of them academic centers >201 beds. The majority of all centers are FACT (Foundation of Accreditation for the Cellular Therapy) accredited, CIBMTR reporting centers & research centers. Sixty-two percent report an individual designated for quality assurance. Approximately half of these institutions have a designated program manager. The predominant APN responsibilities include the following: initial consults (58%), initial H & P (41%), communicate with referring centers (74%), initial contact for complications (45%), daily patient rounds (62%), consulted for BMT pts in PICU (58%), and 75% are involved in clinical decision making on patient care. The predominant APN tasks are orders pre BMT evaluation 62%, prescribes admission orders 50%, performs Bone Marrow aspirate/biopsy 41%, lumbar punctures 33%, infuses stem cells 45%, harvest 50%, manages drug levels 62%, provides discharge education 70%, organizes home care set up and writes discharge orders/prescriptions 50%, & formulates post-BMT follow up plan 70%. The support staff at institutions includes 1-2 physicians (41%), 1-2 nurse practitioners (45%), nurse coordinators (87%), 1-2 data managers (79%), and all have a search coordinator. The median salary ranges from $70,000 -$79,000 with 87% employed full time. Ninety-one percent of respondents identified attending outside institutional meetings and generating research based nursing projects.

Conclusion: Based on the submitted surveys the PBMTC APN is a highly experienced and motivated nurse who has multiple responsibilities over the transplant trajectory. Support for this PBMTC APN deserves further attention related to the vast responsibilities these individuals perform.

Table 1 *Demographic Data*

Demographics	Results
Age	40 - 45 yrs
Sex	Female = 100%
Averge years as RN	> 16 yrs = 58%
Averge years as APN	6 - 10 yrs = 25% , >11yrs =38%
Highest degree	MSN = 70%
Job title	Nurse Practitioner = 37%, Nurse Coordinator = 41%, Clinical Nurse Specialist = 16%
Practice Area	Both in-patient & outpatient care areas = 83%

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ONCE-DAILY INTRAVENOUS BUSULFAN VERSUS ORAL BUSULFAN IN CHILDREN PRIOR TO STEM CELL TRANSPLANTATION: STUDY OF PHARMACOKINETICS AND CLINICAL OUTCOMES

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Background: Dose targeting (IV or oral) of busulfan has been suggested to be associated with higher "alive and engrafted" (A&E) rates due to less graft-failure and lower toxicity. Busulfan is usually given in 4 doses. In this study we compared the outcome of haematopoietic stem cell transplantation (SCT) between once daily IV dose-targeted busulfan (IVdtBU: based on therapeutic drug monitoring (TDM)) and current practice of un-targeted oral busulfan (POBU) and we studied the feasibility of TDM of busulfan in clinical practice.

Methods: Since 2003 IVdtBU was introduced gradually: at first only in pts receiving an SCT for an "inborn error of metabolism", followed by immunodeficiency pts and malignancies. Busulfan was part of a myeloablative regimen. Serotherapy was given in case of an unrelated donor. Pts received a 3-hour infusion of busulfan at a first dose of 120mg/m2 (≥1year) or 80mg/m2 (<1year), or POBU